



March 12, 2019

Senator Mary Abrams, Co-Chair  
Legislative Office Building, Room 3002  
Hartford, CT 06106

Representative Jonathan Steinberg, Co-Chair  
Legislative Office Building, Room 3004  
Hartford, CT 06106

VIA EMAIL: [phtestimony@cga.ct.gov](mailto:phtestimony@cga.ct.gov)

Dear Chairwoman Abrams, Chairman Steinberg and Public Health Committee members:

I write on behalf of Biogen regarding HB 7282, *An Act Concerning Newborn Screening for Spinal Muscular Atrophy (SMA)*, which would add screening for the neurodegenerative disease to Connecticut's newborn screening panel. In parallel, the FY 2020 – FY 2021 Biennium Governor's Budget provides funding to add three nationally recommended disorders for which Connecticut does not currently test – including SMA – to the state's newborn screening panel, and stipulates that the additional operating cost will be offset by revenues generated from increasing the newborn screening fee from \$110 to approximately \$113.

Biogen was founded in 1978 as one of the world's first global biotechnology companies. The company, which has its global headquarters in Cambridge, MA, discovers, develops and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases. Biogen today has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first and only approved treatment for spinal muscular atrophy and is focused on advancing neuroscience research programs in Alzheimer's disease and dementia, MS and neuroimmunology, movement disorders, neuromuscular disorders, acute neurology, neurocognitive disorders, pain and ophthalmology. Biogen also manufactures and commercializes biosimilars of advanced biologics.

SMA is a rare neurodegenerative disease characterized by degeneration of alpha motor neurons in the spinal cord with progressive muscle atrophy, weakness and paralysis. SMA is the most common monogenetic cause of infant mortality. It includes a wide range of phenotypes that are classified into clinical groups on the basis of age of onset and maximum motor function achieved: very weak infants unable to sit unsupported (type 1), non-ambulant patients able to sit independently (type 2), up to ambulant patients with childhood (type 3) and adult onset SMA (type 4).

Epidemiology studies have shown that Type 1 SMA is the most common form of this devastating disease. Natural history studies have shown that infants with Type 1 SMA rarely survived beyond two years of age without requiring permanent respiratory support. These affected infants typically begin to experience symptoms between birth and 6 months of age. Their medical care was typically palliative in nature, aimed at helping them to breathe, cough, and eat. They never achieved motor milestones such as sitting unassisted, standing, and walking.

Just over two years ago, on December 23, 2016, the U.S. Food and Drug Administration approved the first-ever therapy for SMA, now called SPINRAZA, developed by Biogen. Early in the development of SPINRAZA, given the rapidly progressing nature of SMA, we developed a scientific hypothesis that a treatment for SMA would be *most* effective if administered early in the disease progression. At that point in time, we had no idea if SPINRAZA would ever be safe or effective for people living with SMA, but we believed that *if* it worked, newborn screening would be the most efficient way for babies with this disease to have the chance to live their best life.

For the last several years, Biogen – along with the Centers for Disease Control and Prevention, Cure SMA, the Muscular Dystrophy Association, families affected by SMA, and others – have been working together to see SMA added to newborn screening panels. During that time, with the support of CureSMA, Biogen and the CDC developed and validated a cost-effective newborn screening assay for SMA that can be multiplexed with SCID, established and supported pilots in New York and Taiwan to demonstrate that SMA newborn screening works, and opened a clinical trial to test our hypothesis that beginning to treat babies affected by SMA at or near birth would result in meaningfully better outcomes compared to waiting until symptom onset to begin treatment.

Babies with SMA who receive SPINRAZA after symptom onset appear to achieve more motor milestones and live longer without permanent respiratory support than babies who do not receive SPINRAZA. Babies genetically diagnosed with SMA and treated with SPINRAZA before symptoms appear to achieve even greater improvement as reflected in data from our ongoing open label study (NURTURE) in patients who initiated treatment with SPINRAZA prior to the onset of symptoms. For the most recent, public data comparing outcomes for pre-symptomatically vs post-symptomatically treated infants as presented at the 2018 Annual Congress of the World Muscle Society, see Appendix 1 for a figure illustrating the achievement of motor milestones across clinical studies in infants with SMA. The green and blue lines reflect data from infants who initiated treatment prior to symptom onset.

Now that a treatment option is available, and data indicate that initiation of treatment in the pre-symptomatic period improves clinical outcomes, we urge the Connecticut legislature to add SMA to the state's newborn screening panel as expediently as possible. Any delay to implement newborn screening for SMA will almost certainly result in avoidable disability and unnecessary suffering for Connecticut babies with SMA and their families. Recognizing this, New Hampshire, Rhode Island, Vermont, as well as other states across the country have already added newborn screening of SMA to their respective state panels.

Should you have any questions, please contact me at [wildon.farwell@biogen.com](mailto:wildon.farwell@biogen.com) or 617-914-1267.

Sincerely,

Wildon Farwell, MD, MPH  
Senior Director, Clinical Development

CC: Adrienne Manning  
NBS Program Division Director  
Connecticut State Department of Public Health

# HINE Motor Milestone Scores Over Time Across Studies

